Reflecting on muscular dystrophy awareness week

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This past week was muscular dystrophy awareness week, which seems like a short amount of time to focus on such a heartbreaking disease. One in every 3500 boys in the US develops that debilitating and fatal Duchenne muscular dystrophy (DMD) - the most common and serious form of muscular dystrophy - and there is no cure.

CIRM funds a few awards to researchers studying the muscle stem cells called satellite cells. These dot the muscle fibers, ready to spring to action when there's damage. In kids with MD, those satellite cells can't repair the damage and the muscles eventually waste away.

Here's a list of CIRM-funded projects that could lead to new insights or therapies for MD. One Early Translational II project to Michele Calos at Stanford University is especially interesting. Starting in mice, she's proposing to reprogram cells from animals with MD, fix the defective gene, then grow those cells into muscle stem cells that can be transplanted back into the mice. If the technique works, she and her team hope to start working with human cells.

As with all early research there are a lot of unknowns. Can they actually fix the gene? Can they grow up enough muscle stem cells for transplantation? Will those manipulated cells thrive and be able to repair the damaged muscle? And a big question: How on earth do you get those genetically altered cells to all the wasted muscles in the body?

Hopefully in future muscular dystrophy awareness weeks we'll be able to answer some of those questions, and one day if all goes well we'll be writing about a cure.

- A.A.

Tags: Early Translational, muscular dystrophy, Stanford University, calos